

fight^sma

International Advisory Council

Tom Hayes
News Anchor
CFTO TV, Toronto

Robert Meyer
Supervising Producer
CBS, *The Early Show*

Dick Robertson
President
Warner Brothers
Domestic Distribution

Edward Ryan, MD
Toronto West Fertility Associates

Scientific Advisory Committee

CO-CHAIRS:
Christian Lorson, PhD
University of Missouri

Alex E. MacKenzie, MD, PhD
University of Ottawa
Children's Hospital of Eastern Ontario

Elliot J. Androphy, MD
University of Massachusetts
Medical School

Nicholas Boulis, MD
Emory School of Medicine

Walter Bradley, MD
University of Miami

Arthur Burghes, PhD
Ohio State University

Kenneth H. Fischbeck, MD
National Institute of Neurological
Disorders and Stroke

Susan T. Iannaccone, MD, FAAN
University of Texas-Southwestern
Medical Center
Texas Scottish Rite Hospital for Children

Douglas Kerr, MD, PhD
Biogen IDEC

Adrian R. Krainer, PhD
Cold Spring Harbor Laboratory

Robert T. Leshner, MD
University of California San Diego
Rady Children's Hospital

Harold G. Marks, MD
St. Christopher's Hospital for Children

Robert G. Miller, MD
California Pacific Medical Center

Barry S. Russman, MD
Oregon Health and Science University
Shriners Hospital for Children-Portland

June 3, 2013

The Honorable Fred Upton
Chairman
Committee on Energy and Commerce
United States Congress
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Henry Waxman
Ranking Member
Committee on Energy and Commerce
United States Congress
2125 Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton and Ranking Member Waxman:

FightSMA is pleased to offer its endorsement of H.R. 2019, the Kids First Research Act of 2013. We are grateful for the House Energy and Commerce committee's steadfast commitment to pediatric medical research, including the passage earlier this year of H.R. 225, the National Pediatric Research Network Act (NPRNA), and we look forward to committee consideration of H.R. 2019 in the coming weeks. FightSMA is a non-profit organization of families across the nation working to find a treatment for spinal muscular atrophy (SMA), the leading genetic killer of children under the age of two, through enhanced research conducted by our nation's finest medical institutions.

H.R. 2019 would direct resources currently received by presidential campaigns and conventions towards a 10-year pediatric research initiative administered by the National Institutes of Health (NIH). These funds are desperately needed by the pediatric research community and will help address our nation's underinvestment in scientific research for pediatric disorders compared to ailments faced by the adult population.

FightSMA has long believed that the best method for finding a treatment for SMA and other rare pediatric disorders is a collaborative approach to research, including not only cooperation and data sharing across multiple research sites but also between NIH institutes. This model is one that has been utilized in the past with great success by the National Cancer Centers and the Rare Disease Clinical Research Network, and we believe the Kids First legislation's emphasis on collaboration between multiple national research institutes and centers will continue to build on this success. FightSMA is very encouraged by the fact that

Spinal Muscular Atrophy (SMA) kills more babies than any other genetic disease.

fight^sma

International Advisory Council

Tom Hayes
News Anchor
CFTO TV, Toronto

Robert Meyer
Supervising Producer
CBS, *The Early Show*

Dick Robertson
President
Warner Brothers
Domestic Distribution

Edward Ryan, MD
Toronto West Fertility Associates

Scientific Advisory Committee

CO-CHAIRS:
Christian Lorson, PhD
University of Missouri

Alex E. MacKenzie, MD, PhD
University of Ottawa
Children's Hospital of Eastern Ontario

Elliot J. Androphy, MD
University of Massachusetts
Medical School

Nicholas Boulis, MD
Emory School of Medicine

Walter Bradley, MD
University of Miami

Arthur Burghes, PhD
Ohio State University

Kenneth H. Fischbeck, MD
National Institute of Neurological
Disorders and Stroke

Susan T. Iannaccone, MD, FAAN
University of Texas-Southwestern
Medical Center
Texas Scottish Rite Hospital for Children

Douglas Kerr, MD, PhD
Biogen IDEC

Adrian R. Krainer, PhD
Cold Spring Harbor Laboratory

Robert T. Leshner, MD
University of California San Diego
Rady Children's Hospital

Harold G. Marks, MD
St. Christopher's Hospital for Children

Robert G. Miller, MD
California Pacific Medical Center

Barry S. Russman, MD
Oregon Health and Science University
Shriners Hospital for Children-Portland

Page 2 of 2

H.R. 2019 would complement the goals of the NPRNA, a bill our families across the nation strongly support. The NPRNA would establish a national network of research consortia focused on treating rare pediatric diseases. Your leadership and dedication led to the NPRNA's overwhelming House passage earlier this year. We are advocating for quick Senate passage of the NPRNA and look forward to working with you to bring the bill across the finish line as the committee begins the important work of advancing the Kids First Research Act.

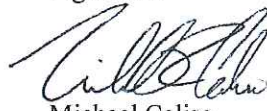
Our children are our most important priority, and FightSMA has been proud to work with the Energy and Commerce committee to enhance our nation's investment in pediatric disease research in a challenging fiscal environment. The advances made over the past decade have been tremendous, and further research over the next ten years will have an even greater impact with the enactment of H.R. 225 and H.R. 2019.

On behalf of FightSMA, thank you again for your leadership in the effort to find treatments and cures for the most devastating diseases that affect our nation's children.

Sincerely,



Daniel Hayden
Executive Director
FightSMA



Michael Calise
Chairman
FightSMA

Spinal Muscular Atrophy (SMA) kills more babies than any other genetic disease.